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UCSF AIDS Research Institute
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Founder & Vice President, Cure Autism Now
Oswald Steward, Ph.D.
Chair & Director-
Reeve, Irvine Research Center, UC Irvine

SB 1565: OPPOSE UNLESS AMENDED

July 10, 2008

Dear Senator Kuehl:

Thank you for the opportunity to comment upon the potential amendments to Senate Bill No. 1565. While the Independent Citizens' Oversight Committee (the "ICOC"), the governing board of the California Institute for Regenerative Medicine ("CIRM"), has taken a position in opposition to the bill on the grounds that is it premature and unnecessary, we appreciate your willingness to engage in a dialogue regarding the potential amendments.

Section 1 of the bill, which would remove the two-thirds vote requirement for funding "vital research opportunities" was added in early June and it has caused a powerful reaction of unanimous opposition from CIRM's governing board. **Proposed subparagraph (E) makes clear that the goal of Section 1 is to eliminate the priority that Proposition 71 places on human embryonic stem cell research. As long as Section 1 remains in the bill, we must strongly oppose SB 1565.**

At a time when opponents of stem cell research are arguing that recent developments obviate the need for human embryonic stem cell research, a position we believe to be incorrect, the proposed amendment to Proposition 71 would send the wrong message to Californians and to the nation at large. It would also thwart the will of the more than seven million Californians who voted for Proposition 71 in order to address the federal funding gap for human embryonic stem cell research, a gap that continues to exist to this day. **By removing the two-thirds vote requirement, the amendment would undermine the very purpose of Proposition 71 – to provide a priority for funding human embryonic stem cell research.** Finally, eliminating the two-thirds vote requirement would be inconsistent with the requirement that Proposition 71 may only be amended to further its purposes. For all of these reasons, which are discussed in greater detail in the attached addendum, we are strongly opposed to the removal of the two-thirds vote requirement.

With respect to Section 2 of SB 1565, we share your view that California state and local government purchasers should have access, at the lowest possible price, to the therapies and drugs derived from CIRM-funded research. Indeed, our regulations include provisions very similar to those set forth in SB1565. Given the complexities of our healthcare system and

the uncertainty regarding the types of therapies and drugs that will be developed as a result of CIRM-funded research, we must retain the flexibility to address issues specific to particular diseases and particular therapies. We appreciate your offer of alternative language, including a waiver process. However, we offer our suggestions below in an effort to reduce the risk of unintended consequences. If you are willing to remove the amendment to the two-thirds vote requirement and to accept our proposed amendments, we would be willing to consider taking a "neutral" position on the bill.

Proposed Addition of Subdivision (e)

We believe it would be preferable to give CIRM greater discretion to establish a waiver mechanism pursuant to the Administrative Procedure Act. The addendum to this letter addresses this issue in more depth. This would permit CIRM to assess changes in medical technology and in the health care sector prior to defining the scope and contours of the waiver and it would provide an opportunity for the Legislature and the public to comment upon the proposed waiver mechanism before it is adopted. Therefore, rather than trying to anticipate the circumstances pursuant to which a waiver may be justified, we recommend replacing subdivision (e) with the following language:

(e) ***Notwithstanding subdivision (c), CIRM may waive the requirement for grantees, and licensees of the grantee, to sell drugs that are, in whole or in part, the result of research funded by CIRM, at one of the three benchmark prices in CalRx, based on a finding that a waiver is necessary to protect the health of Californians whose lives or quality of life is at risk. CIRM shall adopt a regulation or regulations pursuant to the Administrative Procedure Act to implement the waiver provided in this subdivision after notifying the Legislature and conducting a public hearing.***

Proposed Amendments to Subdivision (c)(1)

Your proposed amendments to subdivision (c)(1) clarify what we understand to be the original intent of SB 1565. We believe that further refinements, however, may sharpen the expression of the Legislature's intent. For example, we understand that you intend SB 1565 to apply only to therapies or drugs purchased in California by California state or local government funded programs. The current language, however, would appear also to apply to federally funded programs, including programs funded and administered entirely by the federal government without regard to need. Similarly, we are concerned by the provision that specifies that CalRx, as it exists on January 1, 2008, shall apply regardless of any subsequent changes in the law. While we share your concern about the unintended consequences that could flow from designating a successor program, we believe these concerns could be addressed by incorporating a successor program only if it covers CIRM stem cell-derived therapies or drugs. We therefore propose the following changes to subdivision (c)(1):

> (c)(1) Any plan subject to subdivision (a) shall include a requirement that each grantee and any licensee of the grantee that sells drugs that are, in whole or in part, the result of research funded by CIRM shall sell those drugs ***in California to ~~publicly~~ California state and local government funded programs ~~in California~~*** at one of the three benchmark prices in the California Discount Prescription Drug Program (Division 112 (commencing with Section 130500)), as it exists on January 1, 2008***, or a successor program to the extent that the program applies to California Institute for Regenerative stem cell-derived therapies and drugs***.

Conclusion

CIRM is committed to working with the Legislature to address the important issues raised by SB 1565 and to ensure that Californians have access to therapies and drugs derived from CIRM-funded research. Placing these provisions in statute, however, may hinder our efforts rather than help, because we cannot anticipate all of the challenges we will face in the future.

We recognize that the Legislature could amend the law in the future through urgency legislation, but we are concerned about the potential political opposition to changes that may be required to ensure that Californians have access to a therapy derived from human embryonic stem cells. Given the 70 percent vote requirement in Proposition 71, such opposition could prevent the Legislature from passing an amendment that is essential to ensure access. CIRM's ability to amend its regulations pursuant to the Administrative Procedure Act, on an emergency basis if necessary, does not pose the same risk.

While well-intentioned, SB 1565 is premature and unnecessary. Nonetheless, if you are willing to amend the bill to remove Section 1 and provide for a waiver directive regarding the public pricing policy as described above, we are prepared to recommend a neutral position to the ICOC.

We appreciate your support of CIRM and your willingness to work with us to address these critical issues.

Sincerely,

Robert N. Klein	Alan O. Trounson	Edward E. Penhoet
Chairman, ICOC	President	Vice –Chairman, ICOC

Priority for Funding Human Embryonic Stem Cell Research and Elimination of the Two-Thirds Vote Requirement to Fund Other Stem Cell Research

Proposition 71 arose from Californians' frustration with the federal government's unwillingness to provide adequate funding for the urgent research needed to develop stem cell therapies to treat and cure diseases and serious injuries. In 2001, President Bush issued an executive order to prohibit federal funding for human embryonic stem cell research, with a narrow exception for research involving embryonic stem cell lines derived prior to the issuance of the executive order. As a result, there has been very little federal funding for human embryonic stem cell research.

Proposition 71 was designed to close this gap in federal funding, a gap which persists to this day, by placing a priority on funding for human embryonic stem cell research. Indeed, Proposition 71 expressly states that it "will close this funding gap by establishing an institute which will issue bonds to support stem cell research, *emphasizing pluripotent and progenitor cell research* . . ." (Prop. 71, § 2.) To implement this intent, Proposition 71 creates a priority for research involving pluripotent and progenitor stem cell research that is not receiving timely or sufficient federal funding. (Health & Saf. Code, § 125290.60(c)(1)(C).) Proposition 71 authorizes the funding of other research only if two-thirds of the Grants Working Group recommend the proposal. (*Id*., § 125290.60(c)(1)(D).)

SB 1565 proposes to eliminate the priority that Proposition 71 places on funding for human embryonic stem cell research by removing the two-thirds vote requirement to fund other stem cell research. This proposal comes at a time of a national debate regarding the continuing need for human embryonic stem cell research. Opponents of this research have seized on recent scientific advances to argue that human embryonic stem cell research is no longer necessary, a position with which CIRM and the scientific community strongly disagree. The proposal to eliminate the priority for human embryonic stem cell research in California would send the wrong message to Californians and to the nation at large.

Eliminating the two thirds vote requirement would also undermine the will of the more than seven million California voters who approved Proposition 71 for the express purpose of closing the federal funding gap for human embryonic stem cell research. While we hope that a new administration and 60 supporting votes in the U.S. Senate (to avoid debate being blocked) will authorize federal funding for human embryonic stem cell research, that hope has not yet been realized. It is critical that we respect the determination of California voters to place a priority on funding human embryonic stem cell research.

Finally, the proposed amendment would violate the requirement in Proposition 71 that Proposition 71 may only be amended to further its purposes. (Prop. 71, § 8.) Given the express intent of Proposition 71 to place a priority on funding for human embryonic stem cell research, an amendment that eliminates this priority would fly in the face of the voters' intent and violate the terms of Proposition 71 itself.

CONTINUING CONCERNS REGARDING SB 1565
Waiver Language Cannot Anticipate All Reasons in the Future

We greatly appreciate Senator Kuehl's efforts to address our concerns regarding the potential unintended consequences that could flow from placing a rigid pricing mechanism in statute. Assuming we reach agreement to strike Section 1 of SB 1565 as amended June 9, 2008, we are prepared to work on resolving our opposition to Section 2.

We offered as examples of the need for flexibility and the authority to waive the pricing policy proposed in SB 1565 only based in part on the case of orphan diseases as examples of how a strict application of the CalRx program to drugs and therapies derived in whole or in part from CIRM-funded research could affect our ability to ensure that therapies are available to the patients who most desperately need them. Pricing mechanisms need to be designed to reflect the patient population the therapy needs to reach. While we are in total agreement that California public purchasers should receive at least the same level of pricing as the lowest available commercial rate, we nonetheless believe that we need flexibility to adapt pricing mechanisms to fit the patient population waiting for the therapy as the facts present themselves in each case:

Alzheimers disease:
The age of onset for Alzheimers disease varies. Most patients are diagnosed later in life, when they are often already covered by the Medicare program. But there is a group of "early onset" patients diagnosed in their 40s and 50s where health insurance availability can be extremely problematic. These specific patients have to wait for two years to be eligible for Medicare. Our concern about the pricing mandate of SB 1565 is that it does not allow for a pricing adjustment to fit a subset of patients such as those afflicted by early onset Alzheimers.

Batten's disease:
Current stem cell research aimed at this orphan disease has already presented serious financial challenges to the company involved in attempting to develop a therapeutic product for this disease which today results in the death of young children. If pricing plays a role in the financing solution for that therapeutic product, we need to be able to make such a decision. On balance, bringing the therapeutic product to market outweighs the incremental savings which would result from a one-size-fits-all pricing policy. For example, what if a health care provider helps fund part of the clinical trials for this disease or some other orphan disease and receives special commercial pricing in exchange?

We have a continuing need to be creative in recruiting private funding and stretching California's extremely valuable research dollars. It is very important that we are able to partner with companies who are willing to work with our policies in order to deliver the mission of CIRM and provide affordable treatments of serious diseases to all Californians.

These are only two examples of which we are aware today where the policy in SB 1565 would not allow for customization to the specific facts of the case. There are other factors that can influence pricing decisions as well. As the members of the California Legislature know better than most, ensuring access in our current health care system is an enormously complex challenge. When coupled with nascent and cutting edge medical research, the challenges posed by trying to anticipate and address all of the issues that could arise in the future are insurmountable. For that reason, the ICOC determined that SB 1565 is premature and voted on June 27, 2008 unanimously to oppose it.

Indeed, Senator Kuehl's recent efforts to address our concerns underscore the complexity of trying to anticipate and address these issues before they arise. For example, the proposed language recognizes that some uninsured individuals will not qualify for any public program. However, it is possible that individuals who receive government funded health care or who are privately insured may not qualify for treatment due to a cap on coverage or may be unable to obtain access to a specialist or team of specialists who provide the therapy due to reimbursement rates.

By attempting to specify the precise conditions pursuant to which the ICOC could grant a waiver, the proposed amendments will undoubtedly fail to address other situations in which a waiver would be equally justified. Again, our concern is that the only way these shortcomings could be addressed would be by future legislation, and given the 70 percent threshold for amendments to Proposition 71, it may prove impossible to change the law to accommodate a therapy derived from human embryonic stem cells.

Therefore, we believe it would be preferable to give CIRM a directive to establish a waiver mechanism pursuant to the Administrative Procedure Act. This would permit CIRM to assess changes in medical technology and in the health care sector prior to defining the scope and contours of the waiver and it would provide an opportunity for the Legislature and the public to comment upon the proposed waiver mechanism before it is adopted. Rather than trying to anticipate all of the circumstances pursuant to which a waiver may be justified, we recommend replacing subdivision (e) of your staff's recent draft amendments with the following language:

(e) ***Notwithstanding subdivision (c), CIRM may waive the requirement for grantees, and licensees of the grantee, to sell drugs that are, in whole or in part, the result of research funded by CIRM, at one of the three benchmark prices in CalRx, based on a finding that a waiver is necessary to protect the health of Californians whose lives or quality of life is at risk. CIRM shall adopt a regulation or regulations pursuant to the Administrative Procedure Act to implement the waiver provided in this subdivision, after notifying the Legislature and conducting a public hearing.***